

**AMENDMENT IN THE NATURE OF A SUBSTITUTE
TO H.R. 717**

OFFERED BY MR. BILIRAKIS

Strike all after the enacting clause and insert the following:

1 SECTION 1. SHORT TITLE.

2 This Act may be cited as the “Muscular Dystrophy
3 Community Assistance, Research and Education Amend-
4 ments of 2001”, or the “MD–CARE Act”.

5 SEC. 2. FINDINGS.

6 Congress makes the following findings:

7 (1) Of the childhood muscular dystrophies,
8 Duchenne Muscular Dystrophy (DMD) is the
9 world’s most common and catastrophic form of ge-
10 netic childhood disease, and is characterized by a
11 rapidly progressive muscle weakness that almost al-
12 ways results in death, usually by 20 years of age.

13 (2) Duchenne muscular dystrophy is genetically
14 inherited, and mothers are the carriers in approxi-
15 mately 70 percent of all cases.

16 (3) If a female is a carrier of the dystrophin
17 gene, there is a 50 percent chance per birth that her
18 male offspring will have Duchenne muscular dys-



1 trophy, and a 50 percent chance per birth that her
2 female offspring will be carriers.

3 (4) Duchenne is the most common lethal ge-
4 netic disorder of childhood worldwide, affecting ap-
5 proximately 1 in every 3,500 boys worldwide.

6 (5) Children with muscular dystrophy exhibit
7 extreme symptoms of weakness, delay in walking,
8 waddling gait, difficulty in climbing stairs, and pro-
9 gressive mobility problems often in combination with
10 muscle hypertrophy.

11 (6) Other forms of muscular dystrophy affect-
12 ing children and adults include Becker, limb girdle,
13 congenital, facioscapulohumeral, myotonic,
14 oculopharyngeal, distal, and Emery-Dreifuss mus-
15 cular dystrophies.

16 (7) Myotonic muscular dystrophy (also known
17 as Steinert's disease and dystrophia myotonica) is
18 the second most prominent form of muscular dys-
19 trophy and the type most commonly found in adults.
20 Unlike any of the other muscular dystrophies, the
21 muscle weakness is accompanied by myotonia (de-
22 layed relaxation of muscles after contraction) and by
23 a variety of abnormalities in addition to those of
24 muscle.



1 (8) Facioscapulohumeral muscular dystrophy
2 (referred to in this section as “FSHD”) is a neuro-
3 muscular disorder that is inherited genetically and
4 has an estimated frequency of 1 in 20,000. FSHD,
5 affecting between 15,000 to 40,000 persons, causes
6 a progressive and severe loss of skeletal muscle
7 gradually bringing weakness and reduced mobility.
8 Many persons with FSHD become severely phys-
9 ically disabled and spend many decades in a wheel-
10 chair.

11 (9) FSHD is regarded as a novel genetic phe-
12 nomenon resulting from a crossover of subtelomeric
13 DNA and may be the only human disease caused by
14 a deletion-mutation.

15 (10) Each of the muscular dystrophies, though
16 distinct in progressivity and severity of symptoms,
17 have a devastating impact on tens of thousands of
18 children and adults throughout the United States
19 and worldwide and impose severe physical and eco-
20 nomic burdens on those affected.

21 (11) Muscular dystrophies have a significant
22 impact on quality of life—not only for the individual
23 who experiences its painful symptoms and resulting
24 disability, but also for family members and care-
25 givers.



1 (12) Development of therapies for these dis-
2 orders, while realistic with recent advances in re-
3 search, is likely to require costly investments and in-
4 frastructure to support gene and other therapies.

5 (13) There is a shortage of qualified research-
6 ers in the field of neuromuscular research.

7 (14) Many family physicians and health care
8 professionals lack the knowledge and resources to
9 detect and properly diagnose the disease as early as
10 possible, thus exacerbating the progressiveness of
11 symptoms in cases that go undetected or
12 misdiagnosed.

13 (15) There is a need for efficient mechanisms
14 to translate clinically relevant findings in muscular
15 dystrophy research from basic science to applied
16 work.

17 (16) Educating the public and health care com-
18 munity throughout the country about this dev-
19 astating disease is of paramount importance and is
20 in every respect in the public interest and to the
21 benefit of all communities.



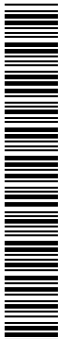
1 **SEC. 3. EXPANSION, INTENSIFICATION, AND COORDINA-**
2 **TION OF ACTIVITIES OF NATIONAL INSTI-**
3 **TUTES OF HEALTH WITH RESPECT TO RE-**
4 **SEARCH ON MUSCULAR DYSTROPHY.**

5 Part A of title IV of the Public Health Service Act
6 (42 U.S.C. 281 et seq.) is amended by adding at the end
7 the following:

8 **“SEC. 404E. MUSCULAR DYSTROPHY; INITIATIVE THROUGH**
9 **DIRECTOR OF NATIONAL INSTITUTES OF**
10 **HEALTH.**

11 “(a) EXPANSION, INTENSIFICATION, AND COORDINA-
12 TION OF ACTIVITIES.—

13 “(1) IN GENERAL.—The Director of NIH, in
14 coordination with the Directors of the National In-
15 stitute of Neurological Disorders and Stroke, the
16 National Institute of Arthritis and Musculoskeletal
17 and Skin Diseases, the National Institute of Child
18 Health and Human Development, and the other Na-
19 tional Institutes of Health Institutes as appropriate,
20 shall expand and intensify programs of such Insti-
21 tutes with respect to research and related activities
22 concerning various forms of muscular dystrophy, in-
23 cluding Duchenne, myotonic, facioscapulohumeral
24 muscular dystrophy (referred to in this section as
25 ‘FSHD’) and other forms of muscular dystrophy.



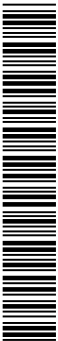
1 “(2) COORDINATION.—The Directors referred
2 to in paragraph (1) shall jointly coordinate the pro-
3 grams referred to in such paragraph and consult
4 with the Muscular Dystrophy Interagency Coordi-
5 nating Committee established under section 6 of the
6 MD–CARE Act.

7 “(3) ALLOCATIONS BY DIRECTOR OF NIH.—The
8 Director of NIH shall allocate the amounts appro-
9 priated to carry out this section for each fiscal year
10 among the national research institutes referred to in
11 paragraph (1).

12 “(b) CENTERS OF EXCELLENCE.—

13 “(1) IN GENERAL.—The Director of NIH shall
14 award grants and contracts under subsection (a)(1)
15 to public or nonprofit private entities to pay all or
16 part of the cost of planning, establishing, improving,
17 and providing basic operating support for centers of
18 excellence regarding research on various forms of
19 muscular dystrophy.

20 “(2) RESEARCH.—Each center under para-
21 graph (1) shall supplement but not replace the es-
22 tablishment of a comprehensive research portfolio in
23 all the muscular dystrophies. As a whole, the centers
24 shall conduct basic and clinical research in all forms
25 of muscular dystrophy including early detection, di-



1 agnosis, prevention, and treatment, including the
2 fields of muscle biology, genetics, noninvasive imag-
3 ing, genetics, pharmacological and other therapies.

4 “(3) COORDINATION OF CENTERS; REPORTS.—
5 The Director of NIH—

6 “(A) shall, as appropriate, provide for the
7 coordination of information among centers
8 under paragraph (1) and ensure regular com-
9 munication between such centers; and

10 “(B) shall require the periodic preparation
11 of reports on the activities of the centers and
12 the submission of the reports to the Director.

13 “(4) ORGANIZATION OF CENTERS.—Each cen-
14 ter under paragraph (1) shall use the facilities of a
15 single institution, or be formed from a consortium of
16 cooperating institutions, meeting such requirements
17 as may be prescribed by the Director of NIH.

18 “(5) DURATION OF SUPPORT.—Support for a
19 center established under paragraph (1) may be pro-
20 vided under this section for a period of not to exceed
21 5 years. Such period may be extended for 1 or more
22 additional periods not exceeding 5 years if the oper-
23 ations of such center have been reviewed by an ap-
24 propriate technical and scientific peer review group
25 established by the Director of NIH and if such



1 group has recommended to the Director that such
2 period should be extended.

3 “(c) FACILITATION OF RESEARCH.—The Director of
4 NIH shall provide for a program under subsection (a)(1)
5 under which samples of tissues and genetic materials that
6 are of use in research on muscular dystrophy are donated,
7 collected, preserved, and made available for such research.
8 The program shall be carried out in accordance with ac-
9 cepted scientific and medical standards for the donation,
10 collection, and preservation of such samples.

11 “(d) COORDINATING COMMITTEE.—

12 “(1) IN GENERAL.—The Secretary shall estab-
13 lish the Muscular Dystrophy Coordinating Com-
14 mittee (referred to in this section as the ‘Coordi-
15 nating Committee’) to coordinate activities across
16 the National Institutes and with other Federal
17 health programs and activities relating to the var-
18 ious forms of muscular dystrophy.

19 “(2) COMPOSITION.—The Coordinating Com-
20 mittee shall consist of not more than 15 members to
21 be appointed by the Secretary, of which—

22 “(A) $\frac{2}{3}$ of such members shall represent
23 governmental agencies, including the directors
24 or their designees of each of the national re-
25 search institutes involved in research with re-



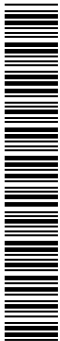
1 spect to muscular dystrophy and representatives
2 of all other Federal departments and agencies
3 whose programs involve health functions or re-
4 sponsibilities relevant to such diseases, includ-
5 ing the Centers for Disease Control and Pre-
6 vention, the Health Resources and Services Ad-
7 ministration and the Food and Drug Adminis-
8 tration and representatives of other govern-
9 mental agencies that serve children with mus-
10 cular dystrophy, such as the Department of
11 Education; and

12 “(B) $\frac{1}{3}$ of such members shall be public
13 members, including a broad cross section of
14 persons affected with muscular dystrophies in-
15 cluding parents or legal guardians, affected in-
16 dividuals, researchers, and clinicians.

17 Members appointed under subparagraph (B) shall
18 serve for a term of 3 years, and may serve for an
19 unlimited number of terms if reappointed.

20 “(3) CHAIR.—

21 “(A) IN GENERAL.—With respect to mus-
22 cular dystrophy, the Chair of the Coordinating
23 Committee shall serve as the principal advisor
24 to the Secretary, the Assistant Secretary for
25 Health, and the Director of NIH, and shall pro-



1 vide advice to the Director of the Centers for
2 Disease Control and Prevention, the Commis-
3 sioner of Food and Drugs, and to the heads of
4 other relevant agencies. The Coordinating Com-
5 mittee shall select the Chair for a term not to
6 exceed 2 years.

7 “(B) APPOINTMENT.—The Chair of the
8 Committee shall be appointed by and be directly
9 responsible to the Secretary.

10 “(4) ADMINISTRATIVE SUPPORT; TERMS OF
11 SERVICE; OTHER PROVISIONS.—The following shall
12 apply with respect to the Coordinating Committee:

13 “(A) The Coordinating Committee shall re-
14 ceive necessary and appropriate administrative
15 support from the Department of Health and
16 Human Services.

17 “(B) The Coordinating Committee shall
18 meet as appropriate as determined by the Sec-
19 retary, in consultation with the chair.

20 “(e) PLAN FOR HHS ACTIVITIES.—

21 “(1) IN GENERAL.—Not later than 1 year after
22 the date of enactment of this section, the Coordi-
23 nating Committee shall develop a plan for con-
24 ducting and supporting research and education on
25 muscular dystrophy through the national research



1 institutes and shall periodically review and revise the
2 plan. The plan shall—

3 “(A) provide for a broad range of research
4 and education activities relating to biomedical,
5 epidemiological, psychosocial, and rehabilitative
6 issues, including studies of the impact of such
7 diseases in rural and underserved communities;

8 “(B) identify priorities among the pro-
9 grams and activities of the National Institutes
10 of Health regarding such diseases; and

11 “(C) reflect input from a broad range of
12 scientists, patients, and advocacy groups.

13 “(2) CERTAIN ELEMENTS OF PLAN.—The plan
14 under paragraph (1) shall, with respect to each form
15 of muscular dystrophy, provide for the following as
16 appropriate:

17 “(A) Research to determine the reasons
18 underlying the incidence and prevalence of var-
19 ious forms of muscular dystrophy.

20 “(B) Basic research concerning the eti-
21 ology and genetic links of the disease and po-
22 tential causes of mutations.

23 “(C) The development of improved screen-
24 ing techniques.



1 “(D) Basic and clinical research for the
2 development and evaluation of new treatments,
3 including new biological agents.

4 “(E) Information and education programs
5 for health care professionals and the public.

6 “(f) REPORTS TO CONGRESS.—The Coordinating
7 Committee shall biennially submit to the Committee on
8 Energy and Commerce of the House of Representatives,
9 and the Committee on Health, Education, Labor, and
10 Pensions of the Senate, a report that describes the re-
11 search, education, and other activities on muscular dys-
12 trophy being conducted or supported through the Depart-
13 ment of Health and Human Services. Each such report
14 shall include the following:

15 “(1) The plan under subsection (e)(1) (or revi-
16 sions to the plan, as the case may be).

17 “(2) Provisions specifying the amounts ex-
18 pended by the Department of Health and Human
19 Services with respect to various forms of muscular
20 dystrophy, including Duchenne, myotonic, FSHD
21 and other forms of muscular dystrophy.

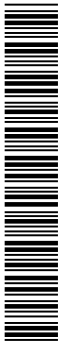
22 “(3) Provisions identifying particular projects
23 or types of projects that should in the future be con-
24 sidered by the national research institutes or other



1 entities in the field of research on all muscular dys-
2 trophies.

3 “(g) PUBLIC INPUT.—The Secretary shall, under
4 subsection (a)(1), provide for a means through which the
5 public can obtain information on the existing and planned
6 programs and activities of the Department of Health and
7 Human Services with respect to various forms of muscular
8 dystrophy and through which the Secretary can receive
9 comments from the public regarding such programs and
10 activities.

11 “(h) AUTHORIZATION OF APPROPRIATIONS.—For the
12 purpose of carrying out this section, there are authorized
13 to be appropriated such sums as may be necessary for
14 each of fiscal years 2002 through 2006. The authorization
15 of appropriations established in the preceding sentence is
16 in addition to any other authorization of appropriations
17 that is available for conducting or supporting through the
18 National Institutes of Health research and other activities
19 with respect to muscular dystrophy.”.



1 **SEC. 4. DEVELOPMENT AND EXPANSION OF ACTIVITIES OF**
2 **CENTERS FOR DISEASE CONTROL AND PRE-**
3 **VENTION WITH RESPECT TO EPIDEMIOLOG-**
4 **ICAL RESEARCH ON MUSCULAR DYSTROPHY.**

5 Part B of title III of the Public Health Service Act
6 (42 U.S.C. 243 et seq.) is amended by inserting after sec-
7 tion 317P the following:

8 **“SEC. 317Q. SURVEILLANCE AND RESEARCH REGARDING**
9 **MUSCULAR DYSTROPHY.**

10 “(a) IN GENERAL.—The Secretary, acting through
11 the Director of the Centers for Disease Control and Pre-
12 vention, may award grants and cooperative agreements to
13 public or nonprofit private entities (including health de-
14 partments of States and political subdivisions of States,
15 and including universities and other educational entities)
16 for the collection, analysis, and reporting of data on
17 Duchenne and other forms of muscular dystrophy. In
18 making such awards, the Secretary may provide direct
19 technical assistance in lieu of cash.

20 “(b) NATIONAL MUSCULAR DYSTROPHY EPIDEMI-
21 OLOGY PROGRAM.—The Secretary, acting through the Di-
22 rector of the Centers for Disease Control and Prevention,
23 may award grants to public or nonprofit private entities
24 (including health departments of States and political sub-
25 divisions of States, and including universities and other
26 educational entities) for the purpose of carrying out epide-



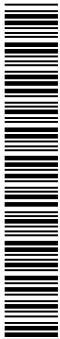
1 miological activities regarding Duchenne and other forms
2 of muscular dystrophies, including collecting and ana-
3 lyzing information on the number, incidence, correlates,
4 and symptoms of cases. In carrying out the preceding sen-
5 tence, the Secretary shall provide for a national surveil-
6 lance program. In making awards under this subsection,
7 the Secretary may provide direct technical assistance in
8 lieu of cash.

9 “(c) COORDINATION WITH CENTERS OF EXCEL-
10 LENCE.—The Secretary shall ensure that epidemiological
11 information under subsections (a) and (b) is made avail-
12 able to centers of excellence supported under section
13 404E(b) by the Director of the National Institutes of
14 Health.

15 “(d) AUTHORIZATION OF APPROPRIATIONS.—There
16 are authorized to be appropriated such sums as may be
17 necessary to carry out this section.”.

18 **SEC. 5. INFORMATION AND EDUCATION.**

19 (a) IN GENERAL.—The Secretary of Health and
20 Human Services (referred to in this Act as the “Sec-
21 retary”) shall establish and implement a program to pro-
22 vide information and education on muscular dystrophy to
23 health professionals and the general public, including in-
24 formation and education on advances in the diagnosis and
25 treatment of muscular dystrophy and training and con-



1 tinuing education through programs for scientists, physi-
2 cians, medical students, and other health professionals
3 who provide care for patients with muscular dystrophy.

4 (b) STIPENDS.—The Secretary may use amounts
5 made available under this section provides stipends for
6 health professionals who are enrolled in training programs
7 under this section.

8 (c) AUTHORIZATION OF APPROPRIATIONS.—There
9 are authorized to be appropriated such sums as may be
10 necessary to carry out this section.

11 **SEC. 6. REPORT TO CONGRESS.**

12 Not later than January 1, 2003, and each January
13 1 thereafter, the Secretary shall prepare and submit to
14 the appropriate committees of Congress, a report con-
15 cerning the implementation of this Act and the amend-
16 ments made by this Act.

Amend the title so as to read: “A bill to amend the
Public Health Service Act to provide for research with re-
spect to various forms of muscular dystrophy, including
Duchenne, Becker, limb girdle, congenital,
facioscapulohumeral, myotonic, oculopharyngeal, distal,
and Emery-Dreifuss muscular dystrophies.”.

